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(FILE 'HOME' ENTERED AT 15:58:38 ON 11 MAY 2009)

FILE 'MEDLINE, CAPLUS, BIOSIS, SCISEARCH, LIFESCI' ENTERED AT 15:59:09 ON
11 MAY 2009

L1 240 S (REDUC? OR DECREAS? OR INHIBIT?) (5A) (GL-3 OR GLOBOTRIAOSYLCER
L2 3 S (AAV OR ADENO-ASSOCIATED(W)VIRUS) (7A) ALPHA-GALACTOSIDASE(W)A
L3 7 S (AAV OR ADENO-ASSOCIATED(W)VIRUS) (7A) ALPHA-GALACTOSIDASE
L4 2 S L1 AND L3
L5 2 DUP REM L4 (0 DUPLICATES REMOVED)

=> d au ti so pi 1-2 15

L5 ANSWER 1 OF 2 CAPLUS COPYRIGHT 2009 ACS on STN
IN Cheng, Seng H.; Meeker, David
TI Combined enzyme replacement, gene therapy and small molecule therapy for
lysosomal storage diseases
SO U.S. Pat. Appl. Publ., 35 pp., Cont.-in-part of U.S. Ser. No. 884,526.
CODEN: USXXCO

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	US 20040204379	A1	20041014	US 2004-758773	20040116
	US 20020095135	A1	20020718	US 2001-884526	20010619
	US 20070280925	A1	20071206	US 2007-762689	20070613

L5 ANSWER 2 OF 2 CAPLUS COPYRIGHT 2009 ACS on STN
AU Takahashi, Hiroshi; Hirai, Yukihiko; Migita, Makoto; Seino, Yoshihiko;
Fukuda, Yuh; Sakuraba, Hitoshi; Kase, Ryoichi; Kobayashi, Toshihide;
Hashimoto, Yasuhiro; Shimada, Takashi
TI Long-term systemic therapy of Fabry disease in a knockout mouse by
adeno-associated virus-mediated muscle-directed gene transfer
SO Proceedings of the National Academy of Sciences of the United States of
America (2002), 99(21), 13777-13782
CODEN: PNASA6; ISSN: 0027-8424

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L5 ANSWER 2 OF 2 CAPLUS COPYRIGHT 2009 ACS on STN
AB Fabry disease is a systemic disease caused by genetic deficiency of a
lysosomal enzyme, α -galactosidase A (α -gal A), and is thought
to be an important target for enzyme replacement therapy. We studied the
feasibility of gene-mediated enzyme replacement for Fabry disease. The
adeno-associated virus (AAV) vector containing the α -gal A gene was
injected into the right quadriceps muscles of Fabry knockout mice. A time
course study showed that α -gal A activity in plasma was increased to
~25% of normal mice and that this elevated activity persisted for
up to at least 30 wk without development of anti- α -gal A antibodies.
The α -gal A activity in various organs of treated Fabry mice
remained 5-20% of those observed in normal mice. Accumulated
globotriaosylceramide in these organs was completely cleared by 25 wk
after vector injection. Redn. of globotriaosylceramide
levels was also confirmed by immunohistochem. and electronmicroscopic
analyses. Echocardiog. examination of treated mice demonstrated structural
improvement of cardiac hypertrophy 25 wk after the treatment. AAV
vector-mediated muscle-directed gene transfer provides an efficient and
practical therapeutic approach for Fabry disease.

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